

Claims:

1. A method of delivering a selected gene to a muscle cell or tissue, said method comprising:

5 (a) providing a recombinant adeno-associated virus (AAV) virion which comprises an AAV vector, said AAV vector comprising said selected gene operably linked to control elements capable of directing the *in vivo* transcription and translation of said selected gene; and

10 (b) introducing said recombinant AAV virion into said muscle cell or tissue.

2. The method of claim 1, wherein said muscle cell or tissue is derived from skeletal muscle.

15 3. The method of claim 1, wherein said muscle cell or tissue is derived from smooth muscle.

20 4. The method of claim 1, wherein said muscle cell or tissue is derived from cardiac muscle.

5. The method of claim 1, wherein said muscle cell is a skeletal myoblast.

25 6. The method of claim 1, wherein said muscle cell is a skeletal myocyte.

30 7. The method of claim 1, wherein said muscle cell is a cardiomyocyte.

8. The method of claim 1, wherein said recombinant AAV virion is introduced into said muscle cell *in vivo*.

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9. The method of claim 1, wherein said recombinant AAV virion is introduced into said muscle cell *in vitro*.

5 10. The method of claim 1, wherein said selected gene encodes a therapeutic protein.

10 11. The method of claim 10, wherein said protein is erythropoietin.

15 12. A muscle cell or tissue transduced with a recombinant AAV virion which comprises an AAV vector, said AAV vector comprising a selected gene operably linked to control elements capable of directing the *in vivo* transcription and translation of said selected gene.

13. The muscle cell of claim 12, wherein said cell is a skeletal myoblast.

20 14. The muscle cell of claim 12, wherein said cell is a skeletal myocyte.

25 15. The muscle cell of claim 12, wherein said cell is a cardiomyocyte.

16. The muscle cell of claim 12, wherein said selected gene encodes erythropoietin.

30 17. A method of treating an acquired or inherited disease in a mammalian subject comprising introducing into a muscle cell or tissue of said subject a therapeutically effective amount of a pharmaceutical composition which comprises (a) a pharmaceutically acceptable excipient; and (b) recombinant AAV virions,
35 wherein said recombinant AAV virions comprise an AAV

vector, said AAV vector comprising a selected gene operably linked to control elements capable of directing the transcription and translation of said selected gene when present in said subject, wherein said introducing is done *in vivo*.

18. A method of treating an acquired or inherited disease in a mammalian subject comprising:

(a) introducing a recombinant AAV virion into a muscle cell or tissue *in vitro* to produce a transduced muscle cell, wherein said recombinant AAV virion comprises an AAV vector, said AAV vector comprising a selected gene operably linked to control elements capable of directing the transcription and translation of said selected gene when present in said subject; and

(b) administering to said subject a therapeutically effective amount of a composition comprising a pharmaceutically acceptable excipient and the transduced muscle cells from step (a).

19. A method for delivering a therapeutically effective amount of a protein systemically to a mammalian subject comprising introducing into a muscle cell or tissue of said subject a pharmaceutical composition which comprises (a) a pharmaceutically acceptable excipient; and (b) recombinant AAV virions, wherein said recombinant AAV virions comprise an AAV vector, said AAV vector comprising a selected gene operably linked to control elements capable of directing the transcription and translation of said selected gene when present in said subject, wherein said introducing is done *in vivo*.

20. A method for delivering a therapeutically effective amount of a protein systemically to a mammalian subject comprising:

(a) introducing a recombinant AAV virion into a muscle cell or tissue *in vitro* to produce a transduced muscle cell, wherein said recombinant AAV virion comprises an AAV vector, said AAV vector comprising a
5 selected gene operably linked to control elements capable of directing the transcription and translation of said selected gene when present in said subject; and

(b) administering to said subject a therapeutically effective amount of a composition
10 comprising a pharmaceutically acceptable excipient and the transduced muscle cells from step (a).

21. An adeno-associated virus (AAV) vector comprising a gene encoding human erythropoietin operably
15 linked to control elements capable of directing the *in vivo* transcription and translation of said gene.

22. A recombinant adeno-associated virus (AAV) virion which comprises the AAV vector of claim 19.
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